Patient-Focused Drug Development Consultation Meeting
October 10, 2012, 2:00 – 3:30 pm
FDA White Oak Campus, Silver Spring, MD
Building 51, Room 1300

Participants

FDA

Patrick Frey	Center for Drug Evaluation and Research (CDER)
Andrea Furia-Helms	Office of Special Health Issues
Jenise Gillespie-Pedersen	CDER
Theresa Mullin	CDER
Gayatri Rao	Office of Orphan Product Development
Paula Rausch	CDER
Andrea Tan	CDER
Graham Thompson	CDER
Robert Yetter	Center for Biologics Evaluation and Research (CBER)

Patient Stakeholders

Jeff Allen	Friends of Cancer Research
Terrell Baptiste	U.S. Hereditary Angioedema Association/Health and Medicine Counsel of Washington
Teresa Barnes	Coalition for Pulmonary Fibrosis
Ronald Bartek	Friedreich's Ataxia Research Alliance
Cynthia Bens	Alliance for Aging Research/Accelerate Cure/Treatments for Alzheimer's Disease
Stacye Bruckbauer	FasterCures/The Center for Accelerating Medical Solutions
Sarah Buchanan	Interstitial Cystitis Association/NephCure Foundation
Meghan Buzby	International Myeloma Foundation
Michelle Carras	Patient Representative
Dane Christiansen	Pulmonary Hypertension Association/Health and Medicine Counsel of Washington
Mary Cathy Collet	Individual patient stakeholder - ALS
Dale Dirks	Health and Medicine Counsel of Washington
Diane Dorman	National Organization for Rare Disorders
Jenny Dwyer	Individual patient stakeholder - ALS
Christin Engelhardt	Pancreatic Cancer Action Network
Edna Fiore	Patient Representative
Ryan Fischer	Parent Project Muscular Dystrophy
Julie Flygare	Individual patient stakeholder - narcolepsy
Eric Gascho	National Health Council
Steve Gibson	ALS Association
Natalie Hamm	American Cancer Society, Cancer Action Network

Lori Hoffman	Sarcoma Foundation of America
Andrea Holka	Attack on Asthma Nebraska
Campbell Hutton	Juvenile Diabetes Research Foundation
Kevin Johnson	ZERO - The End of Prostate Cancer
Scott Johnson	Veterans with ALS
Allison Kassir	King & Spalding LLP
Pamela King	U.S. Hereditary Angioedema Association/Health and Medicine Counsel of Washington
Paul Konanz	Friedreich's Ataxia Parents' Group
Janet Long	U.S. Hereditary Angioedema Association/Health and Medicine Counsel of Washington
Marjana Marinac	Juvenile Diabetes Research Foundation
Mark Mastrofrancesco	Individual patient stakeholder - ALS
Nancy McGrory	New Jersey Chronic Fatigue Syndrome Association
Frank Meeder	National Kidney Foundation
Mishka Michon	Coalition for Pulmonary Fibrosis
ThomasMurphy	Private Citizen and Recent Patient Representative Program Applicant
Martha Nolan	Society for Women's Health Research
MarissaPenrod	Team Joseph/Duchenne Alliance
Carlo Rago	Duchenne Alliance
Teri Robert	Alliance for Headache Disorders Advocacy, American Headache and Migraine Association
Lisa Schlager	FORCE (Facing Our Risk of Cancer Empowered)
RobertaSenzel	Dystonia Medical Research Foundation
Jennifer Sheridan	Parkinson's Action Network
AndrewSperling	National Alliance on Mental Illness
Mark Velleca	Leukemia & Lymphoma Society
Patrick Wildman	ALS Association

Discussion Summary

The meeting began with an overview presentation of FDA's Patient-Focused Drug Development program. FDA described the program's initial development and its short-term and long-term goals: gathering a broader patient perspective on the severity of a particular disease and current treatment options, and the development of a roadmap or model for gathering this type of information. FDA described potential agenda topics for the disease-specific meetings and the questions that could be addressed in order to better understand the patient perspective on this context for regulatory decision-making.

During the meeting, it became clear that some patient stakeholders were under the impression that FDA conducts the research to develop new drugs and biologics. FDA clarified that its role is to provide advice to drug companies who conduct drug development research. Following completion of a clinical

development program, a company then submits a marketing application for the new drug or biologic for FDA review. FDA also emphasized that the list of proposed disease areas does not represent the conditions that are of highest priority in FDA's work. Rather, they are conditions where little is known about the burden of disease in patients or where outcome measures to measure benefit are lacking. FDA believes that the Patient-Focused Drug Development initiative can be particularly helpful in these disease areas. FDA affirmed that the agency reviews applications for drugs in all disease areas, regardless of whether the disease area is discussed as part of Patient-Focused Drug Development.

Several patients and patient advocates asked for more detail on the process of nominating the list of preliminary disease areas. FDA indicated that the preliminary list was developed in collaboration with CDER and CBER and was based on the criteria listed in the Federal Register notice. Numerous suggestions were made regarding expanding the reach of the program. One suggestion was to group similar diseases together in single meetings. Another suggestion was to use surveys or questionnaires to gather responses from larger amounts of patients. Multiple patients and patient advocates requested more accessible physical meeting locations. FDA indicated that remote access would be provided for each meeting. Some also requested an easier method of submitting comments, due to confusion with regulations.gov, and some privacy concerns with commenting to a public docket. FDA notes that comments can be submitted to the docket anonymously by omitting identifying information in relevant fields.

Several participants commented that patients should be the first resource for gathering patient input. The group also discussed the need to ensure that the information that is gathered is usable to FDA. The patient stakeholders expressed support for FDA's efforts to more actively engage patients.